

Cystic Fibrosis Research News

Title:

Strategies used to access CFTR modulators in countries without reimbursement agreements

Lay Title:

How people with CF access CFTR modulators in countries where coverage is not provided by health systems

Authors:

Jonathan Guo¹, Grace Hennessy¹, Benedict Young¹, Andrew Hill²

Affiliations:

¹School of Public Health, Faculty of Medicine, Imperial College London, United Kingdom

²Department of Pharmacology and Therapeutics, University of Liverpool, United Kingdom

What was your research question?

Can people with CF living in countries where CFTR modulator therapies are not funded by health systems access them, and if so by what means?

Why is this important?

CFTR modulators are some of the most expensive oral medications currently available. Their high prices mean that they are out of reach for people with CF, and largely unaffordable for health systems outside of the richest countries in the world. In similar situations in other disease areas patients took it upon themselves to develop alternative methods of accessing treatment. We wanted to explore if similar approaches were being used in CF and whether these could be applied more widely to improve treatment availability around the world.

What did you do?

We carried out a survey of CF doctors in 15 countries where CFTR modulators are not covered by health systems. We then categorised the responses we received based on how they worked, and assessed how many people they were able to benefit as a proportion of the total patient population. We also evaluated how widely applicable and sustainable each method was likely to be in the long term.

What did you find?

People with CF in low- and middle-income countries are taking extraordinary measures at high personal cost to gain access to modulator therapy. The main methods we found were:

Cystic Fibrosis Research News

1. Suing national health systems to provide treatment under human rights laws
2. Travelling abroad to buy cheaper generic drugs out of pocket
3. Receiving drugs as donations.

These strategies were only able to provide benefit to a limited number of patients. They were also unlikely to be widely applicable to large numbers of people or other countries around the world, and unlikely to represent sustainable long-term solutions.

What does this mean and reasons for caution?

It is possible to access CFTR modulator therapies in countries where they are not covered by health systems, however the methods required are generally costly and extremely difficult to pursue for the average person. Our study was limited by the fact that we were only able to speak to a small number of doctors within each country, and that we only surveyed doctors in a small proportion of the countries around the world where CFTR modulators are not available. Therefore, it is unlikely that we have comprehensively identified every method being used around the world.

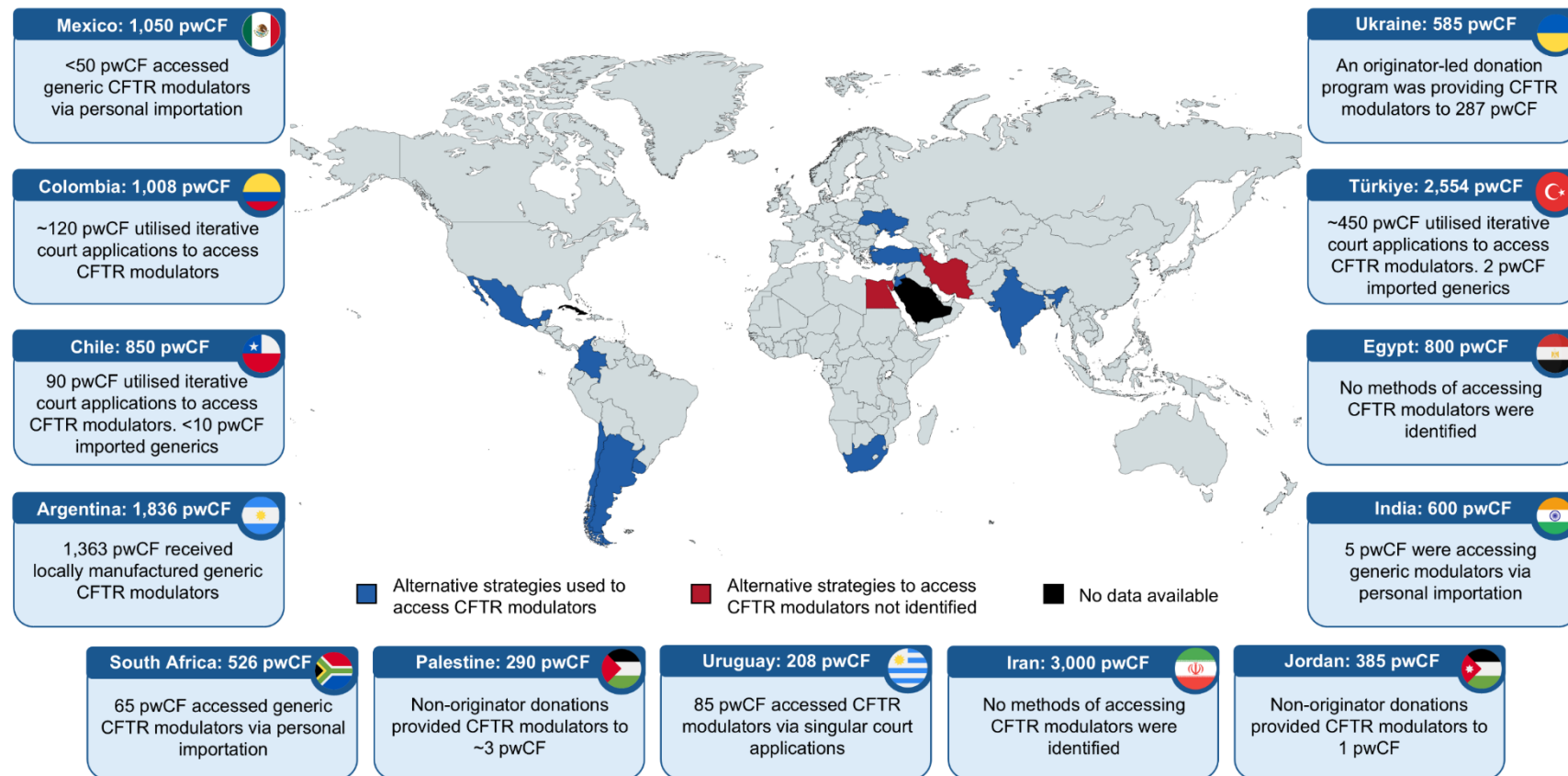
What's next?

Urgent, systemic action is needed to make CFTR modulators more accessible in low- and middle-income countries. Without change, differences in treatment availability will widen existing health gaps between people with CF around the world. More research is needed around treatment access in these areas alongside possible solutions.

Original manuscript citation in PubMed

<https://pubmed.ncbi.nlm.nih.gov/39986975/>

Cystic Fibrosis Research News



Cystic Fibrosis Research News

cfresearchnews@gmail.com